Vertex Reports Third-Quarter 2021 Financial Results

November 2, 2021

-Product revenues of $1.98 billion, a 29% increase compared to Q3 2020-

-Company raises full-year 2021 guidance for product revenues to $7.4 to $7.5 billion-

-Broad pipeline advancing with recent progress marked by unprecedented VX-880 clinical results in T1D; Phase 2 clinical results for VX-147 expected this quarter and for VX-548 in Q1 2022-

BOSTON--(BUSINESS WIRE)--Nov. 2, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the third quarter ended September 30, 2021 and raised full-year 2021 product revenue guidance to $7.4 to $7.5 billion.

"Our financial performance in the third quarter was outstanding, marked by the exceptional continued growth of TRIKAFTA/KAFTRIO. Based on these results, we are again raising our 2021 product revenue guidance, and we see significant additional growth ahead as we continue to deliver this transformational medicine to more people with CF," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "During the last quarter, we also significantly expanded and advanced our pipeline. We announced unprecedented data from the first type 1 diabetes patient dosed with our stem cell-derived, allogeneic islet cells (VX-880). We completed enrollment in the Phase 2 proof-of-concept study of VX-147 in APOL1-mediated FSGS and will report results later this quarter. We also achieved target enrollment in both pivotal CTX001 studies to support our regulatory submissions next year. Finally, we made important advancements in our earlier stage pipeline and expect to submit INDs for both our CF mRNA program and type 1 diabetes cells and device program in 2022."

Third-Quarter 2021 Financial Highlights

	Three Months Ended September 30,		%
	2021	2020	Change
	(in millions, except per share amounts)		
Product revenues, net	$ 1,984	$ 1,536	29%
TRIKAFTA/KAFTRIO	$ 1,556	$ 960	
SYMDEKO/SYMKEVI	$ 81	$ 156	
ORKAMBI	$ 185	$ 226	
KALYDECO	$ 162	$ 194	
GAAP operating income	$ 1,055	$ 672	57%
Non-GAAP operating income	$ 1,188	$ 854	39%
GAAP net income	$ 852	$ 667	28%
Non-GAAP net income	$ 926	$ 697	33%
GAAP net income per share - diluted	$ 3.28	$ 2.53	30%
Non-GAAP net income per share - diluted	$ 3.56	$ 2.64	35%

Product revenues increased 29% compared to the third quarter of 2020, primarily driven by the strong launches of KAFTRIO in Europe and performance of TRIKAFTA in the U.S., including the rapid uptake of TRIKAFTA to children 6-11 years old. Net product revenues in the third quarter of 2021 increased 13% to $1.38 billion in the U.S. and increased 92% to $601 million outside the U.S., compared to the third quarter of 2020.

GAAP and Non-GAAP net income increased compared to the third quarter of 2020, driven by strong product revenue growth.

Cash, cash equivalents and marketable securities were $7.0 billion, an increase of $0.3 billion compared to $6.7 billion as of December 31, 2020. The increase was primarily driven by strong operating cash flow partially offset by repurchases of our common stock authorized under our share repurchase programs and a $900 million payment in the second quarter of 2021 in connection with the amendment of Vertex's collaboration with CRISPR Therapeutics.

Third-Quarter 2021 Expenses

	Three Months Ended September 30,	
	2021	2020
	(in millions)	
Combined GAAP R&D and SG&A expenses	$ 692	$ 678
Combined Non-GAAP R&D and SG&A expenses	$ 561	$ 497
GAAP R&D expenses	$ 494	$ 493
Non-GAAP R&D expenses	$ 403	$ 350
GAAP SG&A expenses	$ 198	$ 185
Non-GAAP SG&A expenses	$ 158	$ 147
GAAP income taxes (1)	$ 231	$ 78
Non-GAAP income taxes	$ 244	$ 155
GAAP effective tax rate (1)	21%	11%
Non-GAAP effective tax rate	21%	18%

Combined GAAP and Non-GAAP R&D and SG&A expenses increased compared to the third quarter of 2020, primarily due to the expansion of Vertex's pipeline in cystic fibrosis and other disease areas and incremental investment to support the launches of Vertex's medicines globally.

GAAP income taxes increased compared to the third quarter of 2020, primarily due to Vertex's increased operating income and the impact of certain discrete tax events (Note 1).

Non-GAAP income taxes increased compared to the third quarter of 2020, primarily due to Vertex's increased operating income.

Full-Year 2021 Financial Guidance

Vertex today increased its full-year 2021 product revenue guidance based on strong year-to-date performance. Vertex's guidance is summarized below:

	Current FY 2021	Previous FY 2021
Product revenues	$7.4 to 7.5 billion	$7.2 to 7.4 billion
Combined GAAP R&D and SG&A expenses (2)	Unchanged	$3.8 to 3.95 billion
Combined Non-GAAP R&D and SG&A expenses (2)	Unchanged	$2.25 to 2.3 billion
Non-GAAP effective tax rate	Unchanged	21% to 22%

Key Business Highlights

Cystic Fibrosis (CF) Marketed Products

Vertex anticipates the number of CF patients treated with our medicines will continue to grow as we enter into additional reimbursement agreements, achieve new approvals for the treatment of younger patients, and expand treatment options for the approximately 10 percent of patients who do not benefit from cystic fibrosis transmembrane conductance regulator (CFTR) modulators, all of which will lead to continued growth of our CF business in the years ahead. Recent progress includes:

- Vertex has signed a Letter of Intent (LOI) with the pan-Canadian Pharmaceutical Alliance (pCPA) regarding the public reimbursement of TRIKAFTA (*elexacaftor/tezacaftor/ivacaftor and ivacaftor*) for eligible patients with CF and has also reached multiple provincial agreements across Canada providing approximately 90% of Canadian patients 12 years of age and older and covered by government insurance with reimbursed access to TRIKAFTA.
- Data are being presented at the North American CF Conference (NACFC) in November from the ongoing 192-week TRIKAFTA open-label extension study which show there has been no loss in mean lung function during long term follow-up at 96 weeks, a first for any CFTR modulator to date. Data from a retrospective study of KALYDECO (*ivacaftor*) will also be presented at the conference. The results showed that people with CF over approximately 6 years old had significantly lower rates of mortality, lung transplant and pulmonary exacerbations compared to those not on treatment.
- Vertex's application for approval of TRIKAFTA in children 6 through 11 years of age has been accepted for priority review by Health Canada.

TRIKAFTA/KAFTRIO is now approved and reimbursed or accessible in more than 20 countries outside the U.S., including Italy, France and Canada.

R&D pipeline

Vertex is delivering on a diversified pipeline of potentially transformative small molecule, cell and genetic therapies aimed at serious diseases. Recent and anticipated progress for key pipeline programs is noted below:

Cystic Fibrosis

- The Phase 3 studies evaluating the new once-daily investigational triple combination of VX-121/*tezacaftor*/VX-561 (*deutivacaftor*) are underway.
- In collaboration with Moderna, Vertex is evaluating CF mRNA therapeutics designed to treat the underlying cause of CF by enabling cells in the lungs to produce functional CFTR protein for the treatment of the 10% of patients who do not produce any CFTR protein. IND-enabling studies are underway and Vertex expects to submit an IND for this program in 2022.

Beta Thalassemia and Sickle Cell Disease (SCD)

- The CTX001 program employs a non-viral *ex vivo* CRISPR gene-editing therapy for the treatment of transfusion-dependent thalassemia (TDT) and severe SCD.
- Data presented to date support the profile of CTX001 as a one-time functional cure for patients with TDT and severe SCD, showing consistent and durable benefit across all treated patients.
- Target enrollment has been achieved in the ongoing clinical trials in TDT and SCD to support the planned regulatory submissions in late 2022.

Type 1 Diabetes (T1D)

- Vertex is evaluating cell therapies designed to replace insulin-producing islet cells that are destroyed in people with T1D with the goal of delivering a potential functional cure.
- VX-880 is a stem cell-derived, allogeneic, fully differentiated, insulin-secreting islet cell replacement therapy, using standard immunosuppression to protect the implanted cells. VX-880 is being evaluated in a Phase 1/2 clinical trial for the treatment of T1D.
- In October, Vertex announced positive Day 90 data for the first T1D patient in the Phase 1/2 clinical trial of VX-880. The patient was treated with a single infusion of VX-880 at half the target dose in conjunction with standard immunosuppressive therapy. The patient demonstrated rapid and robust improvements in multiple measures, including significant increases in fasting and stimulated C-peptide, improvements in glycemic control as measured by HbA1c, and a 91% decrease in exogenous insulin requirements.
- The VX-880 Phase 1/2 study is ongoing at multiple clinical sites in the U.S. and the Clinical Trial Application (CTA) has been approved in Canada.
- Vertex is pursuing a second program in which these stem cell-derived, fully differentiated, insulin-secreting islet cells are encapsulated and implanted in an immunoprotective device. IND-enabling studies are underway and Vertex expects to submit an IND for the cells and device program in 2022.

APOL1-mediated Kidney Diseases (AMKD)

- Vertex is evaluating the potential of oral, small molecule inhibitors of APOL1 function to treat people with AMKD.
- Enrollment is complete in the Phase 2 proof-of-concept study evaluating VX-147 for the treatment of people with APOL1-mediated focal segmental glomerulosclerosis (FSGS) with reduction of proteinuria as the primary endpoint. Results from this study will be reported in the fourth quarter of 2021 and will inform the potential progression of VX-147 into pivotal studies in the broader population of people with APOL1-mediated non-diabetic proteinuric kidney diseases.
- Preclinical data for VX-147 will be presented at the American Society of Nephrology Annual Meeting, November 4-7, 2021.

Pain (NaV1.8)

- Vertex has discovered multiple selective small molecule inhibitors of NaV1.8 with the objective of creating a new class of medicines that have the potential to be highly effective for both acute and chronic pain, without the limitations of opioids and other existing pain medications.
- Two Phase 2 dose ranging acute pain studies with VX-548 are underway, one following bunionectomy surgery and the other following abdominoplasty surgery. Data from the acute pain studies are expected in Q1 2022.

Alpha-1 Antitrypsin (AAT) Deficiency

- Vertex plans to advance one or more novel small molecule zAAT correctors into the clinic in 2022.

Investments in External Innovation

To further expand our capabilities in gene editing, Vertex recently announced two new collaborations.

- In August, Vertex announced a new collaboration with Arbor Biotechnologies Inc. to enhance efforts in developing *ex vivo* engineered cell therapies for multiple serious diseases using Arbor's proprietary CRISPR gene-editing technology.
- In October, Vertex announced a new collaboration with Mammoth Biosciences to develop *in vivo* gene-editing therapies for two diseases using Mammoth's next-generation CRISPR systems.

Non-GAAP Financial Measures

In this press release, Vertex's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP financial results and guidance exclude from Vertex's pre-tax income (i) stock-based compensation expense, (ii) revenues and expenses related to collaborative upfront and milestones payments, including the $900 million upfront payment to CRISPR Therapeutics, and certain other business development activities, (iii) gains or losses related to the fair value of the company's strategic investments, (iv) increases or decreases in the fair value of contingent consideration, (v) acquisition-related costs and (vi) other adjustments. The company's non-GAAP financial results also exclude from its provision for income taxes the estimated tax impact related to its non-GAAP adjustments to pre-tax income described above and certain discrete items. These results should not be viewed as a substitute for the company's GAAP results and are provided as a complement to results provided in accordance with GAAP. Management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding the company's financial position that the company believes is helpful to an understanding of its ongoing business. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, to manage the company's business and to evaluate its performance. The company adjusts, where appropriate, for both revenues and expenses in order to reflect the company's operations. The company's calculation of non-GAAP financial measures likely differs from the calculations used by other companies. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

The company provides guidance regarding combined R&D and SG&A expenses and effective tax rate on a non-GAAP basis. The guidance regarding combined GAAP R&D and SG&A expenses does not include estimates associated with any potential future business development activities. The company does not provide guidance regarding its GAAP effective tax rate because it is unable to forecast with reasonable certainty the impact of excess tax benefits related to stock-based compensation and the possibility of certain discrete items, which could be material.

Vertex Pharmaceuticals Incorporated
Third-Quarter Results
Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Revenues:				
Product revenues, net	$1,984,164	$1,536,271	$5,500,839	$4,575,863
Other revenues	—	2,000	1,000	2,000
Total revenues	1,984,164	1,538,271	5,501,839	4,577,863
Costs and expenses:				
Cost of sales	236,512	186,182	656,813	533,199
Research and development expenses (3)	493,751	493,497	2,356,814	1,362,953
Selling, general and administrative expenses	198,189	184,551	584,935	558,613
Change in fair value of contingent consideration	1,200	1,800	(1,100)	12,600
Total costs and expenses	929,652	866,030	3,597,462	2,467,365
Income from operations	1,054,512	672,241	1,904,377	2,110,498
Interest income	1,116	3,100	3,714	19,919
Interest expense	(15,255)	(13,856)	(46,411)	(41,863)
Other income (expense), net	42,368	84,386	(2,234)	139,621
Income before provision for income taxes	1,082,741	745,871	1,859,446	2,228,175
Provision for income taxes	230,813	78,437	287,456	120,718
Net income	$ 851,928	$ 667,434	$1,571,990	$2,107,457
Net income per common share:				
Basic	$ 3.30	$ 2.56	$ 6.08	$ 8.10
Diluted	$ 3.28	$ 2.53	$ 6.03	$ 7.98
Shares used in per share calculations:				
Basic	257,876	260,392	258,740	260,313
Diluted	259,707	264,079	260,877	264,031

Reconciliation of GAAP to Non-GAAP Net Income and Operating Income
Third-Quarter Results
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
GAAP net income	$ 851,928	$667,434	$1,571,990	$2,107,457
Stock-based compensation expense	102,996	99,539	322,792	332,434
Increase in fair value of strategic investments (4)	(46,679)	(75,750)	(4,993)	(140,866)
Increase (decrease) in fair value of contingent consideration (5)	1,200	1,800	(1,100)	12,600
Collaborative revenues and expenses (6)	26,750	78,050	985,800	141,300
Acquisition-related costs (7)	2,820	2,523	8,460	7,862
Total non-GAAP adjustments to pre-tax income	87,087	106,162	1,310,959	353,330
Tax adjustments (1)	(13,256)	(76,250)	(364,701)	(402,183)
Non-GAAP net income	$ 925,759	$697,346	$2,518,248	$2,058,604
Net income per diluted common share:				
GAAP	$ 3.28	$ 2.53	$ 6.03	$ 7.98
Non-GAAP	$ 3.56	$ 2.64	$ 9.65	$ 7.80
Shares used in diluted per share calculations:				
GAAP and Non-GAAP	259,707	264,079	260,877	264,031

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
GAAP operating income	$1,054,512	$672,241	$1,904,377	$2,110,498
Stock-based compensation expense	102,996	99,539	322,792	332,434
Increase (decrease) in fair value of contingent consideration (5)	1,200	1,800	(1,100)	12,600
Collaborative revenues and expenses (6)	26,750	78,050	985,800	141,300
Acquisition-related costs (7)	2,820	2,523	8,460	7,862
Non-GAAP operating income	$1,188,278	$854,153	$3,220,329	$2,604,694

Reconciliation of GAAP to Non-GAAP Revenues and Expenses
Third-Quarter Results
(in thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
GAAP total revenues	$1,984,164	$1,538,271	$5,501,839	$4,577,863
Collaborative revenues	—	(2,000)	(1,000)	(2,000)
Non-GAAP total revenues	$1,984,164	$1,536,271	$5,500,839	$4,575,863

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
GAAP cost of sales	$ 236,512	$ 186,182	$ 656,813	$ 533,199
Stock-based compensation expense	(1,599)	(1,250)	(4,570)	(3,998)

Non-GAAP cost of sales	$ 234,913	$ 184,932	$ 652,243	$ 529,201
GAAP research and development expenses	$ 493,751	$ 493,497	$2,356,814	$1,362,953
Stock-based compensation expense	(60,995)	(60,770)	(196,412)	(203,732)
Collaborative expenses (6)	(26,750)	(80,050)	(986,800)	(143,300)
Acquisition-related costs (7)	(2,820)	(2,523)	(8,460)	(7,409)
Non-GAAP research and development expenses	$ 403,186	$ 350,154	$1,165,142	$1,008,512
GAAP selling, general and administrative expenses	$ 198,189	$ 184,551	$ 584,935	$ 558,613
Stock-based compensation expense	(40,402)	(37,519)	(121,810)	(124,704)
Acquisition-related costs (7)	—	—	—	(453)
Non-GAAP selling, general and administrative expenses	$ 157,787	$ 147,032	$ 463,125	$ 433,456
Combined non-GAAP R&D and SG&A expenses	$ 560,973	$ 497,186	$1,628,267	$1,441,968

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
GAAP other income (expense), net	$ 42,368	$ 84,386	$ (2,234)	$ 139,621
Increase in fair value of strategic investments (4)	(46,679)	(75,750)	(4,993)	(140,866)
Non-GAAP other (expense) income, net	$ (4,311)	$ 8,636	$ (7,227)	$ (1,245)
GAAP provision for income taxes	$ 230,813	$ 78,437	$ 287,456	$ 120,718
Tax adjustments (1)	13,256	76,250	364,701	402,183
Non-GAAP provision for income taxes (8)	$ 244,069	$ 154,687	$ 652,157	$ 522,901
GAAP effective tax rate	21%	11%	15%	5%
Non-GAAP effective tax rate (8)	21%	18%	21%	20%

Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	September 30, 2021	**December 31, 2020**
Assets		
Cash, cash equivalents and marketable securities	$ 6,960,885	$ 6,658,897
Accounts receivable, net	1,100,372	885,352
Inventories	333,456	280,777
Property and equipment, net	1,042,347	958,534
Goodwill and intangible assets	1,402,158	1,402,158
Deferred tax assets	933,839	882,779
Other assets	845,688	683,311
Total assets	$ 12,618,745	$ 11,751,808
Liabilities and Shareholders' Equity		
Accounts payable and accrued expenses	$ 1,712,855	$ 1,560,110
Finance lease liabilities	558,933	581,476
Contingent consideration	188,500	189,600
Other liabilities	627,749	733,807
Shareholders' equity	9,530,708	8,686,815
Total liabilities and shareholders' equity	$ 12,618,745	$ 11,751,808
Common shares outstanding	256,206	259,890

Notes and Explanations

1: In the three and nine months ended September 30, 2021 and 2020, "Tax adjustments" included the estimated income taxes related to non-GAAP adjustments to the company's pre-tax income and non-recurring discrete benefits to the company's provision for income taxes. The estimated income taxes related to non-GAAP adjustments to the company's pre-tax income included adjustments for (i) stock-based compensation (including an adjustment for excess tax benefits related to stock-based compensation), (ii) changes in the fair value of the company's strategic investments and (iii) collaborative upfront and milestone payments and certain other business development activities and (iv) other adjustments. "Tax adjustments" in the nine months ended September 30, 2021 also included a $100 million discrete benefit related to an increase in the U.K.'s corporate tax rate from 19% to 25%, which was enacted in June 2021 and will become effective in April 2023. "Tax adjustments" in the nine months ended September 30, 2020 also included a $209 million discrete benefit related to the transfer of intellectual property rights to the company's U.K. entity, a $50 million discrete benefit related to the write-off of a long-term intercompany receivable and a $38 million discrete benefit related to an increase in the U.K.'s corporate tax rate from 17% to 19%, which was enacted and became effective in July 2020.

2: The difference between the company's full-year 2021 combined GAAP R&D and SG&A expenses and combined non-GAAP R&D and SG&A expenses guidance relates primarily to $1.0 billion to $1.1 billion of collaborative upfront and milestone payments and certain other business development activities related to existing business development agreements and $415 million to $445 million of stock-based compensation expense. The guidance regarding combined GAAP R&D and SG&A expenses does not include estimates associated with any potential future business development activities.

3: "Research and development expenses" include the $900 million upfront payment to CRISPR in the nine months ended September 30, 2021.

4: "Other income (expense), net" includes net gains related to changes in the fair value of the company's strategic investments and from sales of certain investments.

5: During the three and nine months ended September 30, 2021 and 2020, the change in the fair value of contingent consideration relates to potential payments to Exonics Therapeutics' former equity holders.

6: "Collaborative revenues and expenses" in the three and nine months ended September 30, 2021 and 2020 related to collaborative upfront and milestone payments and certain other business development activities. The company's $900 million upfront payment to CRISPR is included in "Collaborative revenues and expenses" during the nine months ended September 30, 2021.

7: "Acquisition-related costs" in the three and nine months ended September 30, 2021 and 2020 related to costs associated with the company's acquisition of Exonics Therapeutics in 2019.

8: The company released its valuation allowance on the majority of its net operating losses and other deferred tax assets as of December 31, 2018. As of December 31, 2020, the company had utilized substantially all of its remaining federal net operating losses. As a result, a larger portion of the company's tax provision represents a cash tax payable, subject to continued utilization of certain tax credits.

Note: *Amounts may not foot due to rounding.*

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF,Vertex has a robust pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of cell and genetic therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 12 consecutive years on Science magazine's Top Employers list, one of the 2021 Seramount (formerly Working Mother Media) 100 Best Companies, and a best place to work for LGBTQ equality by the Human Rights Campaign. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Kewalramani's statements in this press release, the information provided regarding future financial performance and operations, the section captioned "Full-Year 2021 Financial Guidance" and statements regarding (i) anticipated regulatory filings, data availability, new approvals, and timing thereof, (ii) anticipated future label expansions, (iii) the expectations, development plans and anticipated timelines for the company's medicines, drug candidates and pipeline programs, including study

designs, patient enrollment, data availability and timing thereof, (iv) expectations for the CTX001 collaboration with CRISPR, including anticipated benefits of the collaboration, the potential of CTX001 to be a one-time functional cure for patients with TDT and SCD, and the expectation of regulatory filings next year, (v) expectations for uptake of and expanded access to the company's medicines, including additional reimbursement agreements, (vi) expectations for continued growth in the number of CF patients treated with our medicines, including expansion of treatment options for the patients who do not benefit from CFTR modulators, (vii) expectations for our pain program, including our expectation for available data from the bunionectomy and abdominoplasty trials in the first quarter of 2022, (viii) expectations for an IND submission for our T1D cells and device program in 2022, (ix) plans to advance one or more novel small molecule zAAT correctors into the clinic in 2022, (x) expectations for and anticipated benefits of our collaboration with Moderna to evaluate CF mRNA therapeutics, including our plans to submit an IND for this program in 2022, (xi) our plans to report results from the Phase 2 study evaluating VX-147 in the fourth quarter of 2021 and the potential progression of VX-147 into pivotal studies in a broader population of people, (xii) expectations for our new collaborations with Arbor and Mammoth, and (xiii) expectations for the data presented at the NACFC and the American Society of Nephrology Annual Meeting. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2021 product revenues, expenses and effective tax rates may be incorrect (including because one or more of the company's assumptions underlying its expectations may not be realized), that the company may not be able to submit the anticipated regulatory filings on the expected timeline, or at all, that COVID-19 may have different or more significant impacts on the company's business or operations than the company currently expects, that data from preclinical testing or early clinical trials, especially if based on a limited number of patients, may not be indicative of final results or available on anticipated timelines, that the company may not realize the anticipated benefits from our collaborations with third parties, that data from the company's development programs may not support registration or further development of its potential medicines in a timely manner, or at all, due to safety, efficacy or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com and on the SEC's website at www.sec.gov. You should not place undue reliance on these statements, or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

Conference Call and Webcast

The company will host a conference call and webcast today at 4:30 p.m. ET. To access the call, please dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live and a link to the webcast can be accessed through Vertex's website at www.vrtx.com in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast. An archived webcast will be available on the company's website.

(VRTX-E)

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